

MINUTES

Barth Syndrome Foundation, Inc.

Board of Directors Meeting

November 17, 2022

Members in Attendance:

Andrew Buddemeyer	Board Member
Brandi Dague	Board Member
Emily Milligan	Board Member <i>ex officio</i> , Executive Director
Florence Mannes	Board Member
Jamie Baffa	Board Member, Secretary
Kate McCurdy	Board Member, Chair
Kevin Woodward	Board Member, Treasurer
Mark Greene	Board Member
Maryanne Chrisant, MD	Board Member
Megan Branagh	Board Member
Michelle Florez	Board Member
Miriam Greenberg, PhD	Board Member
Peter van Loo	Board Member

Invited Guests

Bill Belscher	Audit Committee, Chair
Brett Smith	Operations and Communications, BSF
Emily Madalinski	Development and Stewardship, BSF
Erik Lontok, PhD	Director of Research, BSF
Melissa Huang, PhD	Clinical Research Coordinator, BSF
Natalie Cohn	Controller
Steve McCurdy	Finance & Investments Committee

Members in Not Attendance:

B.J. Develle	Board Member
Nina Russell, MD	Board Member

AGENDA

1. *Welcome*
2. *Update: Enzyme Replacement Therapy*
3. *Discussion: New R&D Committee*
4. *Update: Regulatory Affairs*
5. *Update: Giving Tuesday 2022*
6. *Update: Treasurer Report*
7. *Other Business*

The meeting, held via Zoom, was called to order by Kate McCurdy on Thursday, November 17 at 1:01 p.m. ET.

1. Welcome
 - a. Kate McCurdy welcomed board members and invited guests to this additional Board meeting being held in order to complete the agenda from the October Board meeting
2. Update: Enzyme Replacement Therapy
 - a. Steve McCurdy shared an update on Enzyme Replacement Therapy research
 - b. In April 2022, BSF voted to invest \$25,000 in Transcellular Therapeutics (TCT) to cover ERT-related patent costs through the end of 2023
 - c. The next round of funding is coming up soon, and TCT needs a third-party funder to continue to receive NIH funding through an R33 grant (as follow-on beyond his current R61 grant)
 - d. BSF's commitment would be to fund 25% of the total R33 grant amount
 - e. R33 would cover 12 months and have three distinct phases targeted at identifying promising lead compounds for preclinical testing and development
 - f. Jamie Baffa asked if this would come with an additional equity stake in TCT
 - g. Kate, Steve, and Erik shared they are working through this, but it will likely be a direct grant
 - h. Steve then forecasted the proposed conditions under which BSF would fund TCT at a level of \$87,500 and that payments would be tied to key R33 milestones agreed with Michael Chin and TCT, and included in R33 documentation
 - i. Kate reiterated that there is no vote today, but that the board will likely need to make this decision in the coming months
3. Discussion: New R&D Committee
 - a. Kate discussed the challenge of how the board makes decisions about research and development, exemplified by the recent decisions around gene therapy
 - b. Shared that BSF is at a juncture where it would be useful for R&D proposals to be "pre-vetted" by a board Research and Development committee that does not now exist
 - c. The board then reviewed the proposal to create such a group, including rationale and recommended structure of the committee
 - d. Discussion followed
 - e. The board agreed in principle with this idea
 - f. Kate forecasted that the board will likely vote on establishing this committee in December and stand it up shortly thereafter
4. Update: Regulatory Affairs
 - a. There are three distinct regulatory efforts underway:

- i. Supporting Stealth regarding Elamipretide
 - 1. FDA is still engaged and there are still paths forward
 - 2. On another topic, there was an instance where a family was denied compassionate use access to Elamipretide, however, BSF was able to address this with Stealth and the FDA and this issue was resolved
 - ii. BSF serving as an example of an ultra-rare disease for FDA
 - 1. Reenie McCarthy is meeting with Senate staffers this week and members of the community may be asked to write letters to and/or meet with (by phone) their Senators who sit on specific committees
 - iii. In July, BSF had the chance to hold a BSF workshop with the FDA to discuss the challenges of clinical trials with our ultra-rare disease
 - 1. Kate had followed up and expressed that BSF was interested in writing a joint paper with the FDA that outlines a roadmap for Barth syndrome therapy development and possible regulatory pathways forward
 - 2. There are 12 professionals on the project at the moment, including individuals from the FDA, Barth syndrome clinical key opinion leaders (including Hillary Vernon, Todd Cade, and Carolyn Taylor), Kate, Emily Milligan, and James Valentine, JD
 - 3. BSF has committed to outlining a workplan for this effort by December
 - b. Discussion followed
- 5. Update: Giving Tuesday 2022
 - a. Emily Milligan shared current stats for committed donations and fundraisers
 - b. Highlighted that the goal is to build a volunteer network of BSF fundraisers
 - c. BSF is also doing targeted outreach to major donors and those with corporate match programs
 - d. Asked that the board hold their donations until the board match is announced on Giving Tuesday
 - e. Discussion followed
 - f. Emily acknowledged the great work that Emily Madalinski has done for planning Giving Tuesday
- 6. Update: Treasurer Report
 - a. Natalie Cohn reviewed the current financials, noting that BSF is running at an expected deficit of nearly \$400,000 but that the vast majority of revenue will come in between now and the end of the year

- b. Reviewed the current state of strategic investments
- c. Forecasts have been updated, so little change is expected between now and the end of the year, and should serve as the initial basis for 2023 budget
- d. Kate highlighted that BSF's operating results will likely hit even, despite a budgeted deficit, due to expected strategic investments hitting the books in 2023 instead of 2022
- e. BSF is likely to continue to show an unrealized "paper" loss on our investments for the year due to equity and bond market conditions, but we have a conservative portfolio and so are experiencing less decline than the market as a whole
- f. Emily highlighted a generous donation from Barth Italia as well as risks to the BSF budget

7. Adjournment

- a. Kate adjourned the meeting at 2:00 p.m. ET

Respectfully submitted,

Jamie Baffa – Secretary